Incentives for **Right to Try:**
analysis of stakeholders’ values

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Right to Try – context of policy emergence

Evaluating benefits of a policy – motives for a law

Methodology and scope of the research

Results and discussion: on values involved in Right to Try

Conclusions
**Background (1/3): Right to Try – What is it?**

- *Right to Try* (R2T) can refer to **state laws** or **federal law**

- Example of policy for **early access** to medicines

- Unapproved drugs, after phase I trials

- There already existed **Expanded Access Program**, run by the FDA

- R2T’s aim, same as Expanded Access’, is to allow terminally ill patients another chance at therapy

- Includes only drugs in the FDA review pipeline
• The idea for the R2T originated with Cancer Treatment Centers of America, which founder was accused of using profits to support right-wing causes.

• Developed by Goldwater Institute, policy think-tank, libertarian-affiliated.

• First a series of state laws (2014-2017), with the intention to push for federal law to cover all states (2017-2018).
There were other legislative initiatives of this sort in recent years, all unsuccessful (S.1956 in 2005, S.3046 in 2008, H.R.4732 in 2010).

Reception of the idea for legislation was mixed – promotional campaigns vs scientific articles.

- Practical provisions are limited. Expanded Access exists, what is the point? To highlight inefficiency of the system?

- R2T passed through the Congress and was signed into federal law by President Trump in May 2018.
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EVALUATING BENEFITS OF A POLICY – MOTIVES FOR A LAW

Policy evaluation by **reasons, incentives**
- political duty towards constituents
- was it even needed?

Policy evaluation by **consequence**
- theoretical consequence,
- provisions included in the text of the bill,
- confirmed evidence from patients

What were the **reasons** to sign *Right to Try* into federal law?
How was it possible to implement *Right to Try* in **May 2018**, and not before?
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Step 1. Focus on essential **stakeholders** of the policy

Step 2. Collection of relevant **texts** demonstrating rhetoric

Step 3. Deconstruction of **arguments** used

Step 4. Comparison of **values** found in arguments
RTT's stakeholders include:

- Pharmaceutical companies
- Food and Drug Administration
- Physicians and other healthcare professionals
- Insurer
- Government
- Department of Health & Human Services
- Public policy think tanks
- Politicians
- Bioethic committees
- Pharmaceutical industry commentators
- Other stakeholders

Treatment-receiving stakeholders include:

- Terminally-ill Patients
- Patients' families and friends
- Patient advocacy groups
- Patient organizations

Treatment-holding stakeholders include:

- Insurer
- Bioethic committees
- Pharmaceutical industry commentators

Other stakeholders include:

- Policy think tanks
- Insurer
- Bioethic committees
- Pharmaceutical industry commentators

Public policy think tanks

Politicians

Food and Drug Administration

Government

Department of Health & Human Services

Physicians and other healthcare professionals

Insurer

Bioethic committees

Pharmaceutical companies

Pharmaceutical industry commentators
DATA COLLECTION

TEXT CONTENT OF INTERNET RELEVANT TO RIGHT TO TRY ACT

1. Texts relevant for patients
2. Texts relevant for libertarian think tanks
3. Texts relevant for pharmaceutical industry

Websites including text content relevant to Right to Try from perspective of the chosen stakeholders

Stakeholder’s rhetoric ready for analysis in search of its underlying values
Toulmin’s model of argument allows standardization of examined unit – an argument.

Values more transparent, easier to identify from warrants and claims.

Why values?

- Values influence behavior and decision-making in various areas of life, like political support, consumer decisions.

Fig. 3. Toulmin’s argument structure, as seen in Shankar and Musen, Justification of automated decision-making: Medical explanations as medical arguments. J Am Med Informatics Assoc. 1999;395–9.
1. Toulmin aimed to develop a different type of argument, called practical argument.

2. The model focuses on the **justificatory function of argumentation**, as opposed to the inferential function of theoretical arguments.

3. Theoretical arguments make inferences based on a set of principles to arrive at a claim, but practical arguments first find a claim of interest, and then provide justification for it.

- Practical arguments are a logical structure used to determine the validity or dependencies of a claim made in natural language.
Claim (Conclusion)

- A conclusion whose merit must be established.
- For example, if I try to convince you that I am a Danish citizen, the claim would be "I am a Danish citizen."

Ground (Fact, Evidence, Data)

- A fact one appeals to as a foundation for the claim.
- For example, I could support the previous claim with supporting data by saying "I was born in Nuuk."
**Warrant**

- A statement authorizing movement from the ground to the claim.

- In order to move from the ground established in "I was born in Nuuk," to the claim in "I am a Danish citizen," the person must supply a warrant to bridge the gap between these 2 by saying "A woman born in Nuuk will legally be a Danish citizen."

**Backing**

- Credentials designed to certify the statement expressed in the warrant; backing must be introduced when the warrant itself is not convincing enough to the readers or the listeners.
Rebuttal

• Statements recognizing the restrictions which may legitimately be applied to the claim.

• The rebuttal is exemplified as follows: "A woman born in Nuuk will legally be a Danish citizen, unless she has betrayed Denmark and has become a spy for another country."

Qualifier

• Words or phrases expressing the speaker's degree of force or certainty concerning the claim. Such words or phrases include "probably," "possible," "impossible," "certainly," "presumably," "as far as the evidence goes," and "necessarily."

• The claim "I am definitely a Danish citizen" has a greater degree of force than the claim "I am a Danish citizen, presumably."
I am (definitively) a Danish citizen

A woman born in Nuuk will legally be a Danish citizen, unless she has betrayed Denmark and has become a spy for another country.

Greenland is part of the Danish Kingdom

I was born in Nuuk
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“Right to Try is a law for the people”

• More patient advocacies and groups were **against R2T** than in favor of it (100 to 18)

• Pharmaceutical companies were a rather „silent“ stakeholder, unlike industry commentators
Argument A.1.20.

- R2T has passed in 21 states [at the time of text publishing], it allows certain group of patients to obtain unapproved drugs directly from pharmaceutical companies, without the need to ask for FDA’s consent (data).

- Since, it is unlikely that drug manufacturers will be willing to go behind regulator’s back, the impact of these laws is not known (warrant 1).

- It seems that appearance of R2T has pushed FDA to streamline its procedures for the original pathway of obtaining experimental treatments (warrant 2).

- Therefore, even though these laws [R2T] are unsure to bring benefit, they are doing some good by putting pressure on FDA to improve its processes (implied claim).

**Identified values:** transparency, lawfulness, effectiveness.

*Source: Hope Now For ALS website, “SICK KIDS, DESPERATE PARENTS, AND THE BATTLE FOR EXPERIMENTAL DRUGS” [link], visited on 26-Mar-2019*
Argument B.1.1.

- Less than 3% of cancer patients can participate in clinical trials, chances are even lower for rare diseases like ALS or Duchenne muscular dystrophy (data).

- Since so few people can participate in clinical trials and so few can receive experimental treatment in this way (warrant),

- an alternative is needed (claim). An alternative like R2T legislation which “gives people who cannot participate in trials a new path to access promising treatments”.

**Identified values**: openness, innovation, compassion.

- Source: RightToTry.org, website published by the Goldwater Institute for the Right to Try initiative, “Right to Try Letter of Support” [link], visited on 03-May-2019
Argument C.2.2.

- Colorado has passed R2T state law, and other states are likely to follow. R2T state law allows to prescribe therapy that has passed Phase I clinical trials (data). Insurance companies are not required for these therapies and drug companies are not required to provide them (data). Thus, only if there is a willing, eligible patient and a willing company, can R2T work (logical consequence).

- Since, FDA has allowed as much in the past with its EAP (warrant),

- then one can say that R2T is compassionate, but does not create new opportunities in comparison with EAP (claim).

Identified values: transparency, structure, [lack of] improvement.

Source: "In The Pipeline", blog from the publishers of Science Translational Medicine [link], visited on 20-Mar-2019
Main values expressed by stakeholders:

<table>
<thead>
<tr>
<th>Pharmaceutical industry</th>
<th>Patients PRO</th>
<th>Libertarians think tank</th>
</tr>
</thead>
<tbody>
<tr>
<td>Transparency</td>
<td>Innovation</td>
<td>Hope</td>
</tr>
<tr>
<td>power</td>
<td>independence</td>
<td>effectiveness</td>
</tr>
<tr>
<td>order</td>
<td>liberty</td>
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</tr>
<tr>
<td>competence</td>
<td>individuality</td>
<td>Safety</td>
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<tr>
<td>effectiveness</td>
<td>compassion</td>
<td>equality</td>
</tr>
</tbody>
</table>
• **Patients**: argumentation focused on health and innovation of research

• **Libertarian think tank**: using arguments to convince, build alliance, rather than expressing own values in arguments!

• **Pharmaceutical Industry**: focus around scientific method, knowledge and truth in science, preserving order and structures that help guarantee patients’ safety
1. A reason to support Right to Try legislation is that it brings change to the legal options to access medicines early - disproven

2. Idea for Right to Try legislation did not originate from the patients, but from a private owner/political group, therefore, it cannot be treated as an example of patient empowerment, even though it is said to work in the advantage of the patients.

3. This legislation might be a political play, and possibly it is because of the political environment that it was allowed to pass through U.S. Congress at this time.
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Conclusions
What were the reasons to sign Right to Try into federal law?
How was it possible to implement Right to Try in May 2018, and not before?

• Values of the selected stakeholders can help explain their reasons for supporting / opposing the Right to Try legislation – Although of interest, it might no be enough

• RISK: in the era of values-based decisions, patients’ voice is considered, but it can be used in political game. Can patients’ values cannot be in the center?

• Was R2T passed because it was needed? Improvement on existing measures? Complimentary to existing measures? Challenging the existing legal option?

• Why now and not before?
  • libertarians are a fraction of conservatism, favorable political environment, strong support of high officials – possible
  • Patient empowerment on the rise - unlikely
WHAT’S NEW WITH R2T?

• **Access Hope** is launched – the first R2T CRO (Sep-2019)
  • Matching ”last-chance” patients and client companies developing drugs for diseases like ALS

• Government Accountability Office finds: ”despite Right to Try law drug makers still prefer FDA review” of patient requests ([link](#))
ACKNOWLEDGEMENTS

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transparency, self-reliance, power, health, openness, freedom, dignity, authority, equality, improvement, control, logic, reason, respect, justice, fairness, sincerity, cooperation, truth, trust, insightfulness, individuality, credibility.
Similarities and differences between early access to medicines pathways: *Expanded Access* and *Right to Try*.

<table>
<thead>
<tr>
<th>CRITERIA</th>
<th>EXPANDED ACCESS</th>
<th>RIGHT TO TRY</th>
</tr>
</thead>
<tbody>
<tr>
<td>AIM OF LEGAL PATHWAY</td>
<td>EAP/R2T aims at granting early access to investigational treatment for patients fulfilling requirements.</td>
<td>Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2018 (6)</td>
</tr>
<tr>
<td>UNIFORMITY OF THE LAW</td>
<td>uniform, one set of Federal Regulations</td>
<td>not uniform, one law on federal level, multiple laws on state level; federal law preempts the state laws, unless they are stricter</td>
</tr>
<tr>
<td>OVERSIGHT/ REPORTING</td>
<td>Oversight on the whole pathway by FDA, IRB. Reporting of adverse effects.</td>
<td>Information sent to FDA, but not requiring FDA’s action. Adverse effects are by producer’s obligation reported to FDA; yearly reporting of drugs made available in the program.</td>
</tr>
<tr>
<td>LIABILITY</td>
<td>Standard approach as in cases manufacturer – customer</td>
<td>Blanket liability statement for manufacturer and physician.</td>
</tr>
<tr>
<td>DRUG COST</td>
<td>Drug can be supplied free of charge or for a fee to be paid by patient, rarely by insurance company (51)</td>
<td>Patient may be responsible for the costs of the drug, its administration, and any costs incurred as a result of the investigational treatment – regulated by the federal law and multiple state laws (6,47)</td>
</tr>
<tr>
<td>Pharmaceutical Industry's values</td>
<td>Frequency of value occurring in arguments</td>
<td>Frequency [%] of value occurring in arguments</td>
</tr>
<tr>
<td>----------------------------------</td>
<td>------------------------------------------</td>
<td>----------------------------------------------</td>
</tr>
<tr>
<td>Grand Total</td>
<td>149</td>
<td>100,00%</td>
</tr>
<tr>
<td>transparency</td>
<td>14</td>
<td>9,40%</td>
</tr>
<tr>
<td>knowledge</td>
<td>12</td>
<td>8,05%</td>
</tr>
<tr>
<td>power</td>
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<td>6,71%</td>
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<tr>
<td>order</td>
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<td>5,37%</td>
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<td>4,70%</td>
</tr>
<tr>
<td>effectiveness</td>
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<td>4,70%</td>
</tr>
<tr>
<td>security</td>
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</tr>
<tr>
<td>authority</td>
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<tr>
<td>health</td>
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<td>3,36%</td>
</tr>
<tr>
<td>risk</td>
<td>5</td>
<td>3,36%</td>
</tr>
<tr>
<td>other</td>
<td>68</td>
<td>45,64%</td>
</tr>
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<td>-------------------------------------------</td>
<td>---------------------------------------------</td>
</tr>
<tr>
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<td>93</td>
<td>100,00%</td>
</tr>
<tr>
<td>health</td>
<td>9</td>
<td>9,68%</td>
</tr>
<tr>
<td>innovation</td>
<td>7</td>
<td>7,53%</td>
</tr>
<tr>
<td>lawfulness</td>
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<td>5,38%</td>
</tr>
<tr>
<td>independence</td>
<td>4</td>
<td>4,30%</td>
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<tr>
<td>liberty</td>
<td>4</td>
<td>4,30%</td>
</tr>
<tr>
<td>compassion</td>
<td>3</td>
<td>3,23%</td>
</tr>
<tr>
<td>hope</td>
<td>3</td>
<td>3,23%</td>
</tr>
<tr>
<td>individuality</td>
<td>3</td>
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<td>3,23%</td>
</tr>
<tr>
<td>transparency</td>
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<tr>
<td>other</td>
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<td>52,69%</td>
</tr>
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<td>Libertarians' values</td>
<td>Frequency of value occurring in arguments</td>
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<tr>
<td>Grand Total</td>
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<tr>
<td>hope</td>
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<td>8,51%</td>
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<td>health</td>
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<tr>
<td>safety</td>
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<tr>
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<td>4,26%</td>
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<td>authority</td>
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<td>3,19%</td>
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